Understanding Clinical Trials

IKCC
International Kidney Cancer Coalition

Many clinical trials are underway for kidney cancer around the world. For information on finding a clinical trial, contact your local patient organisation or see: www.ikcc.org.

What is a clinical trial?

Every new drug has to be rigorously tested and must pass through pre-clinical research and several "phases" of clinical trials before it can be prescribed to patients. This process can take many years from start to finish.

Clinical trial phases

Pre-clinical phase:
Pre-clinical (prior to the clinic) studies describe all the experiments used to discover and then test a potential drug before it is tested in humans. These experiments will investigate if the treatment kills cancer cells in the lab, if it works in mice, and how it might be expected to work in humans. Once a treatment has passed preclinical testing it might be tested in a clinical trial.

Phase I – Safety and dose. Does it hurt?
Phase I clinical trials, also called "first in human trials", usually recruit a small number of patients. Often times phase I trials are offered to people in whom standard treatments have been tried, but these have failed. Phase I trials are primarily asking the question "does the new treatment hurt anyone? Is it safe?". Phase I trials also investigate how the new treatment works in people, asking how the body breaks the drug down, how much drug is needed in each person, and if there are any side effects.

Phase II – Activity. Does it help?
Phase II clinical trials test the new treatment in a larger group of patients to find out the best treatment schedule. There will usually have been a suggestion of some benefit in a phase 1 trial before a phase II trial is started. The main aim of a phase II trial is to confirm that the new treatment is truly beneficial, as well as addressing important issues such as how often to give the drug, and optimising the dose to be given.

Phase III – Comparison. How does the treatment compare to standard care?
Phase III clinical trials are typically the most uniform clinical trials. They must answer the ultimate question – is the new treatment more effective than the standard treatment? Phase III trials are almost always randomised, often 50:50, such that patients have a 50% chance of receiving the standard treatment and a 50% chance of receiving the new treatment.

If the new treatment proves to be more beneficial than the previous "gold-standard" then the phase III trial is said to be "positive".

More importantly this new treatment now becomes the new "gold-standard" and should change the practice of all doctors around the world treating that disease. If the trial is testing a new drug the company that makes the drug can then apply for approval to licence and sell the drug. Government agencies, such as the US Food and Drug Administration (FDA) or the European Medicines Agency (EMA), approve new treatments if they agree that the drug should be given to patients.

Understanding Clinical Trials

From smaller to larger clinical trials

Phase	I	II	III
Does it…?	Hurt?	Help?	Help much?
Is it…?	Safe?	Active?	Effective?
No. of patients	10-30	30-100	100-300
Pluses	Early access	Promising	Definitely active
Minuses	Safety unknown; could be toxic	Phase 1 could have been wrong	Only 50:50 new drug
Name of the drug	XYZ-123	Unprounouncable-a-nib or –mab	Drugzilla™
Location	One big centre	Few hospitals	World-wide

Phase IV – Real World Data Collection:
Once the new treatment is being used by patients, information is still collected about how well the treatment works in the 'real world' and not in the artificial setting of a clinical trial. This information also shows how the treatment works in the long term.

Patients in the real-world setting often have other illnesses, take other medications, and have real-life issues that may not have been seen in the clinical trial.

Distributed by:

Good to know!

Placebo
A dummy treatment that contains no active drug. It might be a capsule or tablet, it might be a bag of saline. Sometimes just thinking you are being treated can make you feel better. This is a common psychological phenomenon called the "placebo effect".

If you are thinking of taking part in a trial with a placebo group, you need to think about how you will feel if you find out at the end of the trial that you were given the placebo. Some trials give the new treatment to the placebo group after the trial has ended, or swap the treatment and placebo groups during the trial. So even if you are in the placebo group at first, you might still get the new treatment later on. When you ask about a clinical trial, be sure to ask about whether any patients will receive a placebo.

IKCC Factsheet 8: Version 1.0 8/17

IKCC International Kidney Cancer Coalition

The International Kidney Cancer Coalition (IKCC) is an independent international network of patient organisations that focus exclusively, or include a specific focus, on kidney cancer. It is legally incorporated as a Foundation in the Netherlands. **www.ikcc.org info@ikcc.org**